Table e-1. Selected studies on corticosteroids

Reference	Class	Medication	Type of study	No. of treated patients	No. of controls	Duration of treatment	Mean age steroids started, y (SD)	Dropouts greater than 20%	Comparator	Outcome	Adverse events	In prior practice parameter (if yes, what class?)	Reason for change in class from prior practice parameter
12	III	0.9 mg/kg/d of deflazacort	Prospective cohort	30	24	7.2 y (5-8)	8.5 (3.8)	No	Patients not taking medication	Improvement in the rate of development of scoliosis, loss of pulmonary function, and age at loss of ambulation with treatment	Cataracts, weight gain, stress fractures, decreased bone density	No	NA
16	III	2 mg/kg alternating day of deflazacort	RCT	28 patients randomized in a 2:1 scheme, but how many patients were in each group is not clear		2 y	8.0 (1.3)	Yes	Placebo	Improved functional motor scores, age at loss of ambulation, and strength with treatment	Weight gain, behavioral change	Yes (previous Class I)	>20% dropout, no intention to treat, no allocation concealment
4	III	0.75 mg/kg/d 10 days on/10 days off and other undefined dosing of daily and alternate-day prednisone; 0.9 mg/kg/d of deflazacort	Retrospective cohort	Prednisone: n = 16; deflazacort: n = 1	117	Duration of treatment not defined for all; when defined varied from 3- to 264-mo equivalents	4 years and older, not otherwise specified	No	Patients not taking medication	Age at becoming wheelchair bound, requiring part- and full-time noninvasive ventilation, and developing scoliosis by 19 years improves with treatment	Vertebral fractures	No	NA
19	III	0.35 mg/kg/d of prednisolone	RCT, crossover	37 with DMD, 4 with BMD		6 mo on treatment and 6 mo on placebo	7.8 (2.1); range 4.0–10.9	No	Placebo	Improved functional motor scores and strength with treatment	Weight gain	Yes (previous Class I)	No primary outcome identified, no intention to treat or allocation concealment

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23	III	0.75 mg/kg/d of prednisone; 0.9 mg/kg/d of deflazacort	Retrospective cohort	Prednisone: n = 18; deflazacort: n = 12	19	Prednisone: 5.49 y ± 1.98; deflazacort: 5.85 y ± 1.5	Prednisone: 6.9 (1.0); deflazacort: 7.45 (0.97)	No	Patients not taking medication	Improved functional motor scores, strength, FVC, and requirement of surgery for scoliosis with treatment, but no difference between prednisone and deflazacort	Behavioral change, cataracts, excessive weight gain, hypertension, and vertebral fractures	No	NA
17	III	Corticosteroid regimen not specified	Retrospective cohort	291	171	Mean 4.1 (3.4) y	7.4 (2.5)	No	Patients not taking steroids	Delayed onset of cardiomyopathy (fractional shortening) in treated group; age at cessation of ambulation correlated with duration of steroid use	Not reported	No	NA
8	III	0.75 mg/kg/d of prednisone for the first 10 days of each month	RCT, crossover	17		6 mo treatment or placebo, 2 mo washout, 6 mo treatment or placebo	6.29 (0.92)	No	Placebo	Improved functional motor scores and strength with treatment; no change in QoL during treatment compared to placebo	Irritability, cushingoid appearance	No	NA
13	III	0.9 mg/kg/d of deflazacort	Retrospective cohort	30	24	3.2 y ± 1.3	8.4 (2.0)	No	Patients not taking medication	Improved functional motor scores, age at loss of ambulation, strength, pulmonary function, and need for scoliosis surgery with treatment	Short stature, cataracts; no difference in weight gain.	Yes (previous Class I)	Patients were only compared on some potential confounding characteristics, no allocation concealment

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14	III	0.9 mg/kg/d of deflazacort; 0.6 mg/kg/d of deflazacort for the first 20 days of the month	Retrospective cohort	0.9 mg/kg/d: n = 32; 0.6 mg/kg/d for the first 20 days: n = 37	30 and 19, respectively	Not defined (but over 4 years)	Deflazacort 0.9 mg/kg/d: 7.6 (1.6) (range 6–8 y); deflazacort 0.6 mg/kg/d first 20 days: 6.0 (1.5) (range 4–8 y)	No	Patients not taking medication	Improved functional motor scores, strength, and development of scoliosis with treatment (no *p* provided)	Cataracts, fractures	No	NA
5	III	Initiated at 0.9 mg/kg/d of deflazacort without further weight adjustment and reduced for side effects on an individual basis	Retrospective cohort	40	34	5.5 y	7.7 (1.2)	No	Patients not taking medication	Treatment has a positive impact on survival in second decade, loss of ambulation, and pulmonary and cardiac endpoints	Cataracts and short stature, but no difference in weight gain	No	NA
18	III	0.75 mg/kg/d of prednisone; 0.9 mg/kg/d of deflazacort	RCT	Presumably 9 patients in each treatment group	7 historical controls	12 mo	Deflazacort: 8.6 (range 5.3–14.6); prednisone: 7.5 (range 5.1 –10)	No	Natural history controls	Prednisone and deflazacort equally effective in improving muscle strength and functional scores	Hirsutism, cushingoid appearance, weight gain (more with prednisone), cataracts	Yes (previous Class I)	Patients were not compared on baseline characteristics except age and functional score, no intention to treat
25	III	0.75 mg/kg/d of prednisone; 0.9 mg/kg/d of deflazacort	Retrospective cohort	10	25	8.2 y ± 1.14	Not provided	No	Patients not taking medication	Significant improvement in peak cough flow and maximum expiratory pressure	Not reported	No	NA
38	I	0.75 mg/kg/d of prednisolone; 10 mg/kg of prednisone on weekends	RCT	64	64	12 mo	Range 4–10 y; for the 4–6 y age group: weekend dose 5.8 (0.9), daily dose 5.7 (0.7)	No	All patients on treatment	Equivalency in primary strength and safety outcome	Weight gain, cushingoid appearance, behavior change	No	NA

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24	III	1.25 and 2.5 mg/kg of prednisone alternating-day	RCT	1.25 mg/kg: n = 31; 2.5 mg/kg: n = 67	Historical controls	6 mo	Range 5–15 y	No	Placebo	Alternate-day dosing had a less durable benefit for strength and similar side effects compared with daily dosing	Behavior change, weight gain, cushingoid appearance	Yes (previous Class I)	Patients were not compared on baseline characteristics, no intention to treat or allocation concealment
11	II	0.3 and 0.75 mg/kg/d of prednisone	RCT	67 (n = 34 on 0.3 mg/kg/d and n = 34 on 0.75 mg/kg/d)	32	6 mo	Range 5–15 y; prednisone 0.75 mg/kg/d: 9.36 (2.86); predsnione 0.3 mg/kg/d 9.63 (2.53)	No	Placebo	Improved muscle strength with treatment, with a dose response	Weight gain, cushingoid appearance, hirsutism	Yes (previous Class I)	No allocation concealment
33	III	0.3 mg/kg/d and 0.75 mg/kg/d of prednisone; azathioprine added later in study	RCT	n = 33 for 0.3 mg/kg/d; n = 34 for 0.75 mg/kg/d	n = 33 for 0.3 mg/kg/d; n = 34 for 0.75 mg/kg/d	6 mo on steroids and then 12 mo on steroids and azathioprine	Range 5–15 y	No	Placebo	Significant improvement in strength and functional scores	Weight gain, cushingoid appearance, increased blood pressure, short stature	Yes (previous Class I)	Patients were not compared on baseline characteristics, no allocation concealment
26	III	Corticosteroid regimen not specified	Prospective cohort	210 on steroids, 48 with past steroid use	82 not on steroids	Not specified	Range 4–28 y	No	Patients not taking medication	Slower decline in motor and pulmonary function and development of scoliosis in steroid group	Fractures not related to steroid use	No	NA
20	III	0.75 mg/kg/d of prednisolone for 10 days of each month	Retrospective cohort	37	86	Median 1 y (range 2 mo – 9 y)	9.53 (1.2)	No	Patients not taking medication	Positive relationship between duration of treatment with prednisolone and the age at onset of scoliosis but not the severity of scoliosis at the age of 17	Not reported	No	NA

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34	III	0.75 mg/kg/d of prednisone; 0.9 mg/kg/d of deflazacort	Retrospective cohort	Prednisone: n = 36; deflazacort: n = 25; both: n = 14	68	8.04 y (range 0.5–18.5 y)	Not provided	No	Patients not taking medication	Improvement in loss of ambulation, mean degree of scoliosis, and the number of patients with a scoliosis >10°	Vertebral and long bone fractures. increased weight	No	NA
6	III	0.9mg/kg/day of deflazacort	Retrospective cohort	30	24	Mean 15.5 y (treated group) and 14.9 y (nontreated group)	8.5 (3.8)	No	Patients not taking medication	Scoliosis >20°seen in 20% treated group and 92% nontreated group; mortality higher in nontreated group (21% vs 3%)	Cataracts and short stature seen more in treated group; weight change not significant	No	NA
30	III	Undefined dosing of prednisone or deflazacort	Retrospective cohort	48 (29 on prednisone, 19 on deflazacort)	63	3 y ± 2.5	Not provided	No	Patients not taking medication	Lower odds ratio of developing an abnormal shortening fraction with treatment; no difference between prednisone and deflazacort	Noted but not specified	No	NA
31	III	0.75 mg/kg/d of prednisone; 0.9 mg/kg/d of deflazacort	Retrospective cohort	Prednisone: n = 9; deflazacort: n = 5	23	>6 mo	7.5 (0.7)	No	Patients not taking medication	Less likely to develop ventricular dysfunction with treatment	Shorter stature	No	NA
32	III	0.75 mg/kg/d of prednisone for the first 10 days of the month that was switched to 0.9 mg/kg/d of deflazacort as quickly as possible	Prospective cohort	17	17	7–14 y	Median 7 y	No	Patients not taking medication	Improved T2 relaxation times and left ventricular systolic function	Not reported	No	NA
9	II	0.75 and 1.5 mg/kg/d of prednisone	RCT	103	103	6 mo	Prednisone 0.75 mg/kg/d: 9.16 (2.95); prednisone 1.5 mg/kg/d: 9.09 (2.59)	No	Placebo	Improved functional motor scores, strength, and FVC with no significant difference between the treatment groups	Weight gain, cushingoid appearance, and hirsutism; no difference between the 2 regimens	Yes (previous Class I)	No allocation concealment or intention to treat (although dropouts <20%, so does not downgrade further)

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15	III	1 mg/kg/d of deflazacort	RCT	14	14	12 mo	Range 5–11 y	No	Placebo	Improved functional motor scores and strength with treatment	Cushingoid appearance, increased appetite, hirsutism, and behavior change	Yes (previous Class I)	Patients were not compared on baseline characteristics except weight and creatine kinase, no allocation concealment
22	III	0.75 mg/kg/d of prednisolone	RCT	44	23	Not defined, but >2 y	8.83 (1.25)	Yes	Patients not taking medication	Improved functional motor scores, loss of ambulation, strength, and pulmonary function with treatment	Weight gain, cushingoid appearance, infections	No	NA
35	III	0.75 mg/kg/d of prednisone; 0.9 mg/kg/d of deflazacort	Prospective cohort	67	67	3–15 mo	Range 5 y to loss of ambulation	No	All patients on treatment	No significant difference in impact on progression of weakness between the 2 treatments	Weight gain; no difference between prednisone and deflazacort	Yes (previous Class I)	Patients were not compared on baseline characteristics, no allocation concealment
36	III	Daily prednisolone; intermittent prednisolone (on 10 d/off 10 d); alternate-day prednisolone; daily deflazacort; switchers	Prospective cohort	Daily prednisolone: n = 136; intermittent prednisolone: n = 154; alternate-day prednisolone: n = 15; daily deflazacort: n =19; switchers: n = 72	32	4.3 y (range 0.5–7.5); 3.6 y (range 0.5–8.5); 5.0 y (range 2.4–7.5); 4.4 y (range 0.6–7.9); 4.1 y (range 0.7–7.8), respectively	Range 3.4–9.8 y	No	Patients on daily vs intermittent regimen	Age at loss of ambulation earlier in intermittent group (HR 1.57, CI 0.87–2.82); faster decline motor function scale in intermittent group; no difference in respiratory and pulmonary function	More side effects with daily regimen: cushingoid, behavior, hypertension, short stature	No	NA

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7	III	Deflazacort 0.9 mg/kg/d or prednisone 0.5-0.75 mg/kg/d, with either ACE inhibitor or ARB, and calcium and vitamin D	Retrospective cohort	n = 63 on steroids	n = 23 not treated with steroids	11.0 y (4.8)	8.6 (3.5)	?	Patients not on steroids	Less death in steroid group (7/63 vs 10/23, *p* =0.001); survival rate at 15 y greater in treated group (78.6% vs 27.9%, *p* = 0.005); mortality HR for steroids 0.24 (0.07-0.91); less cardiomyopathy in treated group (7/63 vs 14/23, *p* = 0.0001); cardiomyopathy HR for steroids 0.38 (0.16-0.9); slower rate of decline in LVEF in treated group (-0.43% vs -1.09%, *p* = 0.01); slower rate of decline in FS for treated group (-0.32% vs -0.65%, *p* = 0.002)	Short stature in steroid group more frequent (*p* < 0.0001); no difference in weight or hypertension	No	NA
27	III	0.9 mg/kg/d of deflazacort	Retrospective cohort	19; 4 patients had taken prednisone previously	13; and additional historical controls	Years, otherwise not defined for all treated patients; follow-up range 49–79 mo	Not provided	No	Natural history controls as well as patients not on treatment	Improved functional motor scores, strength, and pulmonary function with treatment; no change in cardiac outcome	Short stature, cataract, obesity, fractures	Yes (previous Class I)	While patients were matched for baseline age, there is no quantitative comparison, and there is no comparison of other baseline characteristics, no allocation concealment, no intent to treat, and no allocation concealment

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37	III	0.75 mg/kg of prednisone 10 d on/10 d off; 0.75 mg/kg of prednisone10 d on/20 d off; 0.75 mg/kg/d of prednisone	Retrospective cohort	30;1;1, respectively; n = 33 on prednisone	14	Mean follow-up 57 mo but length of treatment not specified	Median 5 y (range 2.5–8.6)	No	Patients not taking medication	No difference in height and weight between the ages of 4 and 9 y between treated patients and controls	Not reported	No	NA
21	III	0.75 mg/kg alternating-day prednisolone	Prospective cohort	66	22	2.75 y (range 1.5–5)	Not provided	No	Patients not taking medication	Improved age at loss of ambulation and rate of developing scoliosis with treatment	Not reported	No	NA

Abbreviations: ACE = angiotensin-converting enzyme; ARB = angiotensin receptor blocker; BMD = Becker muscular dystrophy; CI = confidence interval; DMD = Duchenne muscular dystrophy; FS = fractional shortening; FVC = forced vital capacity; HR = hazard ratio; LVEF = left ventricular ejection fraction; NA = not applicable; QoL = quality of life; RCT = randomized controlled trial.